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C.2 Medicines for haemoglobinopathies

MSF strongly supports the proposals from the EML Secretariat including the following changes to the listings of medicines in Section 10.3 “Other medicines for haemoglobinopathies” on the WHO Model List of Essential Medicines (EML) and the WHO Model List of Essential Medicines for Children (EMLc):

- Create separate sub-sections for “Medicines for sickle-cell disease” and “Medicines for thalassaemias".
- List hydroxycarbamide (hydroxyurea) in the new sub-section for medicines for sickle-cell disease.
- Exceptionally use the United States Adopted Name (USAN) “hydroxyurea” in preference to the International Non-proprietary Name (INN) “hydroxycarbamide” (with the INN being reported in brackets beside the USAN).
- List deferasirox and deferoxamine in the new sub-section for medicines for thalassaemias.

Hydroxycarbamide (hydroxyurea) is also included on the EML for use in the treatment of chronic myeloid leukaemia (CML). The same change regarding the USAN / INN is proposed for its listing for CML.

Sickle cell disease (SCD) is a genetic disorder that affects the production of hemoglobin, the protein in red blood cells that carries oxygen throughout the body. People with SCD have an abnormal hemoglobin, called hemoglobin S, which can cause red blood cells to become rigid and block blood flow, leading to severe vaso-occlusive crises and other life-threatening complications. SCD is the commonest inherited blood disorder globally. 25 million people have SCD, with the majority (80%) found in Sub-Saharan Africa. Most importantly, the disease is extremely lethal for children in high burden countries. Hydroxycarbamide (hydroxyurea) is originally a drug for chronic myeloid leukemia (CML). In the context of SCD, it increases host production of fetal normal hemoglobin, hemoglobin F, and thus reduces mortality, the incidence and severity of vaso-occlusive crises and improve general outcomes.

In some MSF operations, mainly in Sub-Saharan Africa, patients with SCD are managed within a general cohort of pediatric and adolescent chronic diseases. Patients might be treated exclusively for severe complications or receive preventive care with regular follow-up including specific vaccinations, daily oral prophylactic penicillin, folate supplementation, antimalarial prophylaxis in endemic areas, and recently hydroxycarbamide (hydroxyurea). MSF has been using hydroxycarbamide in its programs since 2020. The MSF cohort with regular follow-up comprises about 2000 children and adolescents. MSF expects to provide hydroxyurea to at least 1000 patients in 2025-2026 and will prescribe this medicine using selection criteria defined in a consensus meeting with African experts.

Therefore, MSF strongly supports the creation of sub-sections for “Medicines for sickle-cell disease” and “Medicines for thalassaemias” and to list hydroxycarbamide (hydroxyurea) in the new sub-section for medicines for sickle-cell disease.

Based on the mapping of possible suppliers of hydroxyurea, MSF found that both the United States Adopted Name (USAN) ‘hydroxyurea’ and the International Nonproprietary Name (INN)

'hydroxycarbamide' are used. In everyday language and also scientific publications, "hydroxyurea" is more readily use, therefore MSF supports to use the United States Adopted Name (USAN) "hydroxyurea" in preference to the International Nonproprietary Name (INN) "hydroxycarbamide".

MSF would like to draw the attention of the Expert Committee to the following facts:

- Regarding access to care: in the remote areas where MSF operates, despite the availability of effective interventions for early diagnosis and management, there is a limited access to comprehensive care for people with SCD, and particularly to hydroxyurea.

- Regarding hydroxyurea: Many affordable generics of hydroxyurea 500mg capsules, (developed for CML) exist in high-income countries but are off label for SCD; until recently, very few options of hydroxyurea with a proper indication for treatment of SCD, and more specifically, low strength hydroxyurea, were available.

- New suppliers of hydroxyurea appeared recently, targeting specifically SCD. In 2024, MSF performed a mapping of possible suppliers and found the following results: there were many available suppliers of hydroxyurea 500mg, two suppliers of hydroxyurea 100mg scored tablets (i.e. 50mgx2) and of hydroxyurea 1000mg scored tablets (i.e. 250mgx4), two suppliers of hydroxyurea oral suspension, and two suppliers of hydroxyurea 100mg capsules. MSF notices that only the solid oral dosage forms of hydroxyurea are included in the EML. Oral liquid forms are not included and should be considered for inclusion.

- Regarding the listing of deferasirox and deferoxamine in the new sub-section for medicines for thalassaemias: Deferasirox and deferoxamine are indicated for the treatment of iron overload in adult and paediatric patients with anaemia requiring repeated blood transfusion. MSF agrees that deferasirox and deferoxamine are mainly used in patients with thalassaemia syndromes, but some patients with SCD can require repeated blood transfusion and iron chelation. Therefore, MSF suggests listing also deferasirox and deferoxamine in subsection 10.3.1 "Medicines for sickle-cell disease".

MSF recommends that, to help improve sustained access to hydroxyurea, WHO should convene a dialogue with ministries of health, treatment providers, patient groups, and manufacturers to work towards pooled forecasting, coordinated procurement, and preferential prices for LMICs with a high burden of SCD.

MSF urges the 25th Expert Committee on the Selection and Use of Essential Medicines to accept all the proposals from the EML Secretariat and to take into consideration the proposal of inclusion of oral liquid form of hydroxyurea and the proposal of listing deferasirox and deferoxamine in subsection 10.3.1 "Medicines for sickle-cell disease".

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